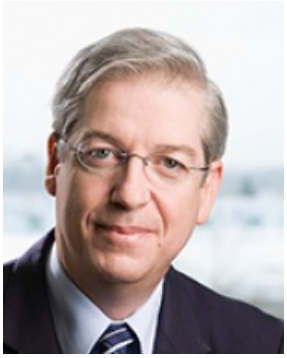
CIRM Funded Clinical Trials

A Phase 1 Study of ECT-001 Expanded Cord Blood and Myeloablative Regimen with Reduced Toxicity in Patients with Severe Sickle Cell Disease

Disease Area:	Sickle Cell Disease
Investigator:	Pierre Caudrelier
Institution:	ExCellThera Inc.
CIRM Grant:	CLIN2SCD-11674
Award Value:	$2,000,000
Trial Sponsor:	ExCellThera Inc.
Trial Stage:	Phase 1
Trial Status:	Launching
Targeted Enrollment:	N/A



Pierre Caudrelier

Details:
Sickle Cell Disease (SCD) is an inherited blood disorder caused by a single gene mutation that results in the production of “sickle” shaped red blood cells. It affects an estimated 100,000 people, mostly African American, in the US and can lead to multiple organ damage as well as reduced quality of life and life expectancy. Although blood stem cell transplantation can cure SCD fewer than 20% of patients have access to this option due to issues with donor matching and availability.

This trial will use umbilical cord stem cells from healthy donors, which could help solve the issue of matching and availability. In order to generate enough blood stem cells for transplantation, a small molecule will be used to expand these blood stem cells. The product cells, named ECT-001, will then be transplanted into children and young adults with SCD.

Design:
Phase I single-arm, open-label trial

Goal:
Evaluate safety and feasibility

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